CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

CSTONE REPORTS EFFICACY AND SAFETY DATA FROM THE ARROW TRIAL OF PRALSETINIB IN CHINESE PATIENTS WITH ADVANCED RET FUSION-POSITIVE NSCLC AFTER PLATINUM-BASED CHEMOTHERAPY AT IASLC WCLC 2020

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce today that results from a cohort of Chinese patients in the global phase I/II ARROW trial of pralsetinib were presented in an oral presentation at the International Association for the Study of Lung Cancer ("**IASLC**") 2020 World Conference on Lung Cancer ("**WCLC**") hosted by the IASLC. The results showed that pralsetinib has robust and durable anti-tumor activity and a well-tolerated safety profile in patients that enrolled at China sites who had advanced rearranged during transfection ("**RET**") fusion-positive non-small cell lung cancer ("**NSCLC**") previously treated with platinum-based chemotherapy.

Pralsetinib is a selective and potent RET inhibitor developed by CStone's partner Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"). CStone has an exclusive collaboration and license agreement with Blueprint Medicines for the development and commercialization of pralsetinib in greater China, which encompasses mainland of the People's Republic of China, the Special Administrative Region of Hong Kong, the Special Administrative Region of Macau and Taiwan.

The ARROW trial is a global phase I/II clinical study designed to evaluate the safety, tolerability and efficacy of pralsetinib in patients with RET fusion-positive NSCLC, RET-mutant medullary thyroid cancer ("**MTC**") and other advanced solid tumors with RET fusions. Data from the ARROW study showed that pralsetinib had robust and durable anti-tumor activity in multiple advanced RET-altered solid tumors, including RET fusion-

positive NSCLC. For ARROW trial patients with RET fusion-positive NSCLC previously treated with platinum-based chemotherapy, this presentation illustrated that the efficacy and safety results of pralsetinib (400 mg once daily) from China sites were consistent with previously reported data from the global patient population. These results mark the first medical conference presentation of data for pralsetinib in Chinese patients with RET fusion-positive NSCLC previously treated with platinum-based chemotherapy.

As of a data cutoff date of May 22, 2020, a total of 37 patients with advanced RET fusion-positive NSCLC from ten China sites were enrolled in the global ARROW study, and received a starting pralsetinib dose of 400 mg once daily. All patients had received at least one platinum-based chemotherapy regimen, approximately half (49%) had received no less than three types of systemic treatment options, and 32% had received no less than three types of chemotherapy regimens. Tumor response was assessed by blinded, independent central review ("**BICR**") using Response Evaluation Criteria in Solid Tumors.

Efficacy: Pralsetinib showed robust clinical activity in patients with RET fusion-positive NSCLC after platinum-based chemotherapy

- In 32 patients who had measurable disease at baseline per BICR, the confirmed overall response rate was 56%, including one complete response and 17 partial responses ("**PR**"). In addition, two patients achieved PR pending confirmation. The disease control rate reached 97% and 1 patient was not evaluable.
- Among 18 patients with confirmed response, the median time to first response was 1.9 months.
- 89% of confirmed responders have remained on treatment.
- The median duration of response ("**DOR**") was not reached, and the 6-month DOR rate was 83%.
- Responses were observed regardless of RET fusion genotype.

Safety: Pralsetinib was well-tolerated with a manageable safety profile

- Pralsetinib was well-tolerated. There were no adverse events related to treatment with pralsetinib that led to treatment discontinuation or death.

Professor Yi-long Wu of Guangdong Provincial People's Hospital, a principal investigator of the ARROW study said: "The development of RET-targeted therapy is a huge breakthrough in the precision treatment of lung cancer. We are encouraged by the clinical trial results of pralsetinib in Chinese patients with RET fusion-positive NSCLC, and the potential benefits this investigational treatment may bring to them. Currently, clinical research is being conducted in patients with RET fusion-positive NSCLC who have not been previously treated with platinum-based chemotherapy, as well as RET-mutant medullary thyroid cancer and other solid tumors with RET fusions. We hope pralsetinib will be an important new treatment option for more patients in the future."

Professor Qing Zhou, the professor of Guangdong Provincial People's Hospital and presenter for this report, said: "Lung cancer is the most common malignant tumor in China and there is no selective RET inhibitor that has been approved in this country. This study confirmed for the first time the efficacy and safety of pralsetinib in Chinese patients with RET fusion-positive NSCLC previously treated with platinum-based chemotherapy. Pralsetinib has the potential to address unmet medical needs for these patients and may become the first selective RET inhibitor in China."

Dr. Jason Yang, the Chief Medical Officer of CStone, said: "We are very excited about the results; among RET fusion-positive NSCLC patients who have undergone prior treatment, the confirmed overall response rate was 56% and the disease control rate reached 97% and showed durable efficacy, with a manageable safety profile. China's National Medical Products Administration has accepted the new drug application for pralsetinib with a priority review designation. In addition, we plan to release the key results from two first-line studies of pralsetinib in RET fusion-positive NSCLC and RET-mutant medullary thyroid cancer Chinese patients treated without platinum-based chemotherapy this year. If the results are positive, we will submit new drug applications, and we look forward to bringing pralsetinib to Chinese patients as soon as possible."

About Pralsetinib

Pralsetinib is a once-daily oral targeted therapy approved by the United States ("**U.S.**") Food and Drug Administration ("**FDA**") under the brand name GAVRETO™ for the treatment of adult patients with metastatic RET fusion-positive NSCLC as detected by an U.S. FDA approved test, adult and pediatric patients 12 years of age and older with advanced or metastatic RET-mutant MTC, and adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory, if appropriate.

Pralsetinib is not approved for the treatment of any other indication in the U.S. by the U.S. FDA or for any indication in any other jurisdiction by any other health authority.

Pralsetinib is designed to selectively and potently target oncogenic RET mutations, including secondary RET mutations predicted to drive resistance to treatment. In preclinical studies, pralsetinib inhibited RET at lower concentrations than other pharmacologically relevant kinases, including VEGFR2, FGFR2 and JAK2.

Blueprint Medicines and Roche are co-developing pralsetinib globally (excluding greater China) for the treatment of patients with RET-altered NSCLC, thyroid cancer, and other solid tumors. The European Medicines Agency validated a marketing authorization application for pralsetinib for the treatment of RET fusion-positive NSCLC. The U.S. FDA granted breakthrough therapy designation to pralsetinib for the

treatment of RET fusion-positive NSCLC that has progressed following platinum-based chemotherapy and for RET mutation-positive MTC that requires systemic treatment and for which there are no alternative treatments.

About CStone

CStone is a biopharmaceutical company focused on developing and commercializing innovative immuno-oncology and precision medicines to address the unmet medical needs of cancer patients in China and worldwide. Established at the end of 2015, CStone has assembled a world-class management team with extensive experience in innovative drug development, clinical research, and commercialization. The Company has built an oncology-focused pipeline of 14 drug candidates with a strategic emphasis on immuno-oncology combination therapies. Currently, six late-stage candidates are at pivotal trials. CStone's vision is to become a world-renowned biopharmaceutical company that is leading the way to conquering cancer.

For more information about CStone, please visit: www.cstonepharma.com.

By order of the Board
CStone Pharmaceuticals
Dr. Frank Ningjun Jiang
Chairman

Suzhou, People's Republic of China, January 28, 2021

As at the date of this announcement, the Board of Directors of the Company comprises Dr. Frank Ningjun Jiang as Chairman and Executive Director, Dr. Wei Li, Mr. Qun Zhao, Mr. Yanling Cao, Mr. Xianghong Lin and Dr. Lian Yong Chen as non-executive Directors, and Dr. Paul Herbert Chew, Mr. Ting Yuk Anthony Wu and Mr. Hongbin Sun as independent non-executive Directors.